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**Reverse transcriptase inhibitors as a novel therapeutic approach for neurological autoimmune disorders**

**Grant Award Details**

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Reverse transcriptase inhibitors as a novel therapeutic approach for neurological autoimmune disorders

**Grant Type:** Inception - Discovery Stage Research Projects

**Grant Number:** DISC1-08825

**Project Objective:** To validate the L1 RT target in AGS and to explore astrocytes as the source of the inflammatory response in the disease.

**Investigator:**

<b>Name:</b>	Alysson Muotri
<b>Institution:</b>	University of California, San Diego
<b>Type:</b>	PI

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**Disease Focus:** Neurological Disorders

**Human Stem Cell Use:** Embryonic Stem Cell, iPS Cell

**Cell Line Generation:** iPS Cell

**Award Value:** \$210,060

**Status:** Closed

**Grant Application Details**

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**Application Title:** Reverse transcriptase inhibitors as a novel therapeutic approach for neurological autoimmune disorders

**Public Abstract:****Research Objective**

We found that approved anti-retroviral drugs could stop inflammation and block neurodegeneration. We propose to validate the re-purpose efficacy of these clinically-approved retroviral drugs.

**Impact**

We have identified an unexpected cause to a brain inflammation and a potential simple treatment. Our research could help millions of patients affected by a broad range neuro-immunological disorders.

**Major Proposed Activities**

- Determine the specificity of the anti-retroviral drugs to inhibit cellular reverse transcriptase and reduce human neurodegeneration.
- Determine the molecular mechanism responsible for the observed neuronal toxicity.
- Determine the non-cell autonomous component of neuro-inflammation using co-culture cellular assays.

**Statement of Benefit to California:**

Neuroinflammation is an important component of several neurological disorders, including autism, ALS, Parkinson, Alzheimer, lupus, multiple sclerosis and aging. These conditions affects millions of people in California and worldwide. However, little is known about what initiates such an inflammatory process. Our innovative approach is of high clinical relevance, because it suggests that patients suffering with neuroinflammation could immediately benefit from available anti-retroviral drugs.

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